
Modeling brain disease in a dish: really?

Journal:	Cell Stem Cell
Publication Year:	2012
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PubMed link:	22704498
Funding Grants:	Molecular and Cellular Transitions from ES Cells to Mature Functioning Human Neurons, Crosstalk: Inflammation in Parkinson's disease (PD) in a humanized in vitro model

Public Summary:

Cellular programming and reprogramming technology has provided a new way to investigate traits of human development and disease. This technology is particularly useful for diseases in which the affected tissue is not available for cell purification and in which aspects of cell development are crucial for the pathology. The central nervous system (CNS) is a good example of tissue that falls into this category. Modeling human brain diseases using induced pluripotent stem cells or induced neural cells has the remarkable potential to generate insights into understanding disease mechanisms and opening new avenues for clinical intervention. Researchers now have the opportunity to study human disease in living, developing neural cells that carry the disease-specific genetic variants that are present in the patient. In addition, reprogramming technology represents a fresh approach for developing original diagnostic tools and obtaining novel drug candidates for CNS therapy. Importantly, candidate compounds for treating CNS defects fail in clinical trials in over 90% of cases due to poor targeting, lack of efficacy, and unacceptable side effects. We firmly believe that reprogramming technology can offer a valuable additional tool for screening and validating CNS compounds for pharmaceutical companies in the near future.

Scientific Abstract:

Cellular programming and reprogramming technology (CPART) presents a novel approach for understanding disease progression and mechanism. In addition, CPART provides an innovative opportunity for developing diagnostic tools and novel drug candidates for therapy. In this Forum, we will discuss obstacles and solutions for modeling brain disease using CPART.

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